

**Boston Children's Hospital
Translational Research Program (TRP)
Mooney Family Initiative for Translational and Clinical Studies in Rare Diseases**

Please submit all applications to TRP@childrens.harvard.edu.

For questions regarding these instructions, please contact the TRP (trp@childrens.harvard.edu).

1. **Background:** The goal of the Mooney Family Initiative for Translational and Clinical Studies in Rare Diseases award (TCSR D) is to assist investigators in the execution of their early phase human trials (including in particular first in human and pilot/feasibility studies), to allow investigators to more rapidly pursue their research which could ultimately impact the lives of pediatric patients suffering from serious or life-threatening diseases. By providing extraordinary clinical trial support to an investigator, this program seeks to enhance the translation of discovery science such that this science can have a more immediate global impact in rare pediatric diseases. The Translational Research Program (TRP) has created this new initiative to provide support to investigators in three spheres:
 - Clinical trial costs on selected, peer-reviewed protocols.
 - Clinical care costs for patients participating in selected clinical trials who do not have insurance coverage and no means to pay.
 - Transportation and housing cost for families outside NE, including international patients.
2. **Purpose of grant:** The program seeks to enhance the opportunities for pediatric patients to enroll in innovative trials. Funds awarded through the Mooney Family Initiative will provide financial resources to bring families and patients from afar to enroll in selected studies. Additional funds will be awarded to support to costs necessary to enhance and/or speed enrollment into trials.
3. **Funding details:** Funding through the Mooney Family Initiative is intended to offset/supplement the uncovered costs of a trial including but not limited to clinical care and research costs, laboratory testing, specimen acquisition and archiving and correlative biological studies. The award is not intended to cover the investigator's salary expenses or salaries of other study team members. Funding will cover up to 10% of patients enrolled in the specific clinical trial for which the award is made up to \$400K inclusive of 20% overhead. Funds are also available (up to \$100k or current available) to cover a patient's travel, housing and other incidental expenses for patients from outside NE, including international patients. No overhead is awarded with these non-clinical care costs. Requests for these non-clinical expenses can be made in addition to or separately from requests for clinical expenses.
4. **Allowable expenses: NIH allowable costs (OMB A110 cost principles), however salaries are not allowed:**
 - a. Clinical care expenses not covered by insurance or other means of funding.
 - b. Trial expenses including (but not limited to) ancillary labs, specimen acquisition and archiving and correlative biological studies via the TransLab and Biorepository at BCH.
 - c. Non-clinical related expenses:
 - i. Travel—including airfare, Ground Transportation (Taxi, Bus, Airport service), lodging
 - ii. Visa\Passport\Immigration Costs
 - iii. Interpreters Services
 - iv. Food, diapers, formula
 - v. Home Health Care
 - vi. Outpatient Prescriptions
 - vii. Follow-up appointments
5. **Eligibility:** Applications will be accepted from Principal Investigators (PIs) who are full-time faculty of Boston Children's Hospital. At time of application, the trial must be IRB and FDA (if required) approved and the trial must be active and open for enrollment. Peer-reviewed and funded NIH trials will be given priority. While multi-institutional trials, including those in which the BCH faculty is not the overall PI, will

be considered, those trials representing translation of scientific discoveries from BCH, particularly first in human studies, or trials in which BCH is the lead site will have priority.

6. **Process:** Applications will be accepted on a periodic basis and will be reviewed by an external review committee consisting of pediatric academic, translational research and clinical trial experts from outside Boston Children's Hospital.

Review criteria: Applications will be prioritized for funding based on the review panel's evaluation of the following:

1. The likely impact of completion of the trial on child health.
2. The role of BCH basic/discovery and translational research in development of the clinical trial, compelling nature of the need.
3. The track record of the investigative team in carrying out human early phase therapeutic and device trials.

These aspects should be covered in the application.

7. **Signatures:** The signature of the primary investigator (and co-investigator) is required. Additionally, the signature of the primary investigator's Department Chairman or Division Chief's signature is required.
8. **Full applications:** Research plan/proposal (not to exceed 5 pages, including figures but excluding references) should include a summary of the trial and its goal as well as a strong justification for why additional funding is needed and an explanation of how this additional funding will speed enrollment to the trial. A statement that the trial is open for accrual must be included. Proposal text must be limited to five pages (including figures but excluding references).

Additional items to be included as an appendix (not included in 5- page limit) are requested:

- IRB approval letter
- FDA (IND/IDE) approval (if applicable)
- Summary notes from IND/IDE Review Group (IIRG) meeting (if appropriate)
- Current funding for study
- Reviewer comments from grants that fund any part of the trial

Required format: *Full* applications must be submitted in single spaced text, one-half inch margins, and no smaller than an 11-point font. Arial or Helvetica typeface is preferred. The primary applicant's name must appear in the upper right hand corner of each page. Standard NIH forms for abstracts, updated CV, and other support may be used and may be submitted as separate files. Applications must be submitted electronically, including the letter of support from Department Chair/Division Chief.

Send a PDF file of assembled proposal to TRP@childrens.harvard.edu. Application forms (modified from PHS 398) are attached.

9. **Letter of Support:** Full applications must include a letter of support from the primary applicant's chairperson. Included in the letter of support must be a statement regarding the importance of the research to the field and priority of the trial for the department/division, including prioritization of the patient population to be enrolled in the trial. The Chairperson should affirm that the applicant will have the support of the Department/Division to carry out the proposed studies.
10. **Composition of Research Proposal:** Invited research proposals should include:
 - a. Face Page
 - b. Abstracts (scientific and lay)
 - c. Table of Contents
 - d. Detailed Budget (may apply for either or both cost categories listed below:
 - i. Clinical and trial associated expenses

- ii. Non-clinical related expenses
- e. Budget justification
- f. Biosketch(es) (include PI and co-investigator; use PHS 398 form)
- g. Other support (PHS 398 form)
- h. Research Plan (5 page limit), including the following items: The role of BCH basic/discovery and translational research in development of the clinical trial, compelling nature of the need and the track record of the investigative team in carrying out human early phase therapeutic and device trials.
- i. Literature cited
- j. Appendix
 - IRB approval
 - FDA approval
 - Summary notes from IIRG meeting (if appropriate)
 - Current funding outline for study
 - Reviewer comments from grants that fund any part of the trial
- k. Letters of support from Department/Division chairperson
- l. Letters of support from collaborators or consultants*
**optional*